

Neural Stem Cells as a Developmental Candidate to Treat Alzheimer Disease

Grant Award Details

Neural Stem Cells as a Developmental Candidate to Treat Alzheimer Disease

Grant Type: Early Translational I

Grant Number: TR1-01245

Project Objective: The goal of the project is to identify a DC for the treatment of AD. They compared several human NSC transplantation approaches as well as viral delivery of a growth factor into the mammalian brain in order to improve cognitive function.

Investigator:

Name: Frank LaFerla
Institution: University of California, Irvine
Type: PI

Name: Richard Boyd
Institution: Monash University
Type: Partner-PI

Disease Focus: Aging, Alzheimer's Disease, Neurological Disorders

Collaborative Funder: Victoria, Australia

Human Stem Cell Use: Adult Stem Cell, Embryonic Stem Cell

Award Value: \$3,599,997

Status: Closed

Progress Reports

Reporting Period: Year 1

[View Report](#)

Reporting Period: Year 2

[View Report](#)

Reporting Period: Year 3

[View Report](#)

Grant Application Details

Application Title: Neural Stem Cells as a Developmental Candidate to Treat Alzheimer Disease

Public Abstract: Alzheimer disease (AD), the most common cause of dementia among the elderly and the third leading cause of death, presently afflicts over 5 million people in the USA, including over 500,000 in California. Age is the major risk factor, with 5% of the population over age 65 affected, with the incidence doubling every 5 years thereafter, such that 40-50% of those over age 85 are afflicted. Being told that one suffers from AD is one of the most devastating diagnoses a patient (and their family/caregivers) can ever receive, dooming the patient to a decade or more of progressive cognitive decline and eventual loss of all memory. At the terminal stages, the patients have lost all reasoning ability and are usually bed-ridden and unable to care for themselves. As the elderly represent the fastest growing segment of our society, there is an urgent need to develop therapies to delay, prevent or treat AD. If the present trend continues and no therapy is developed, over 16 million Americans will suffer from AD by 2050, placing staggering demands on our healthcare and economic systems. Thus, supporting AD research is a wise and prudent investment, particularly focusing on the power that stem cell biology offers.

Currently, there is no cure or means of preventing AD. Existing treatments provide minor symptomatic relief– often associated with severe side effects. Multiple strategies are likely needed to prevent or treat AD, including the utilization of cell based approaches. In fact, our preliminary studies indicate that focusing on the promise of human stem cell biology could provide a meaningful therapy for a disease for which more traditional pharmaceutical approaches have failed.

We aim to test the hypothesis that neural stem cells represent a novel therapeutic strategy for the treatment of AD. Our broad goal is to determine whether neural stem cells can be translated from the bench to the clinic as a therapy for AD.

This proposal builds on extensive preliminary data that support the feasibility of neural stem cell-based therapies for the treatment of AD. Thus, this proposal focuses on a development candidate for treating Alzheimer disease. To translate our initial stem cell findings into a future clinical application for treating AD, we assembled a world class multi-disciplinary team of scientific leaders from the fields of stem cell biology, animal modeling, neurodegeneration, immunology, genomics, and AD clinical trials to collaborate in this early translational study aimed at developing a novel treatment for AD. Our broad goal is to examine the efficacy of human neural stem cells to rescue the cognitive phenotype in animal models of AD. Our studies aim to identify a clear developmental candidate and generate sufficient data to warrant Investigational New Drug (IND) enabling activity. The proposed studies represent a novel and promising strategy for treating AD, a major human disorder for which there is currently no effective therapy.

Statement of Benefit to California:

Neurological disorders have devastating consequences for the quality of life, and among these, perhaps none is as dire as Alzheimer disease. Alzheimer disease robs individuals of their memory and cognitive abilities, such that they are no longer able to function in society or even interact with their family. Alzheimer disease is the most common cause of dementia among the elderly and the most significant and costly neurological disorder. Currently, 5.2 million individuals are afflicted with this insidious disorder, including over 588,000 in the State of California. Hence, over 10% of the nation's Alzheimer patients reside in California. Moreover, California has the dubious distinction of ranking first in terms of states with the largest number of deaths due to this disorder.

Age is the major risk factor for Alzheimer disease, with 5% of the population over age 65 afflicted, with the incidence doubling every 5 years such that 40-50% of the population over age 85 is afflicted. As the elderly represent the fastest growing segment of our society, there is an urgent need to develop therapies to prevent or treat Alzheimer disease. By 2030, the number of Alzheimer patients living in California will double to over 1.1 million. All ethnic groups will be affected, although the number of Latinos and Asians living with Alzheimer will triple by 2030, and it will double among African-Americans within this timeframe. To further highlight the direness, at present, one person develops Alzheimer disease every 72 seconds, and it is estimated that by 2050, one person will develop the disease every 33 seconds! Clearly, the sheer volume of new cases will create unprecedented burdens on our healthcare system and have a major impact on our economic system. As the most populous state, California will be disproportionately affected, stretching our public finances to their limits. To illustrate the economic impact of Alzheimer disease, studies show that an estimated \$8.5 billion of care were provided in one year in the state of California alone (this value does not include other economic aspects of Alzheimer disease). Therefore, it is prudent and necessary to invest resources to try and develop strategies to delay, prevent, or treat Alzheimer disease now.

California has taken the national lead in conducting stem cell research. Despite this, there has not been a significant effort to utilize the power of stem cell biology for Alzheimer disease. This proposal seeks to reverse this trend, as we have assembled a world class group of investigators throughout the State of California and in [REDACTED] to tackle the most significant and critical questions that arise in translating basic research on human stem cells into a clinical application for the treatment of Alzheimer disease. This proposal is based on an extensive body of preliminary data that attest to the feasibility of further exploring human stem cells as a treatment for Alzheimer disease.

Source URL: <https://www.cirm.ca.gov/our-progress/awards/neural-stem-cells-developmental-candidate-treat-alzheimer-disease>